SEBASTIANSTRONG
FOUNDATION

2023 SEBASTIANSTRONG DISCOVERY SCIENCE AWARD

Treating Rhabdomyosarcoma (RMS) with Novel CAR-T Therapy

Institution: Albert Einstein College of Medicine, New York
Primary Investigator: Xingxing Zang, M.Med., Ph.D., Professor & Louis Goldstein Swan Chair in Cancer Research, Director of Institute for Immunotherapy of Cancer
Co-Investigators: David Loeb, M.D., Ph.D., Professor & Chief, Division of Pediatric Hematology, Oncology, and Cellular Therapy, Children's Hospital at Montefiore, Allison Martin, M.D, Assistant Professor & Co-Director of Adam Gaynes Pediatric Neuro-Oncology Program, Children's Hospital at Montefiore
Type of Childhood Cancer: Rhabdomyosarcoma
Amount Funded: $500,000

With a steadfast commitment to propelling the frontier of pediatric cancer research forward, the SebastianStrong Foundation annually presents the Discovery Science Award to the most promising early-stage research projects. This initiative underscores our resolve to nurture ideas that could one day lead to breakthrough treatments for childhood cancer. This year, our Medical Advisory Board—comprised of leading pediatric oncology experts nationwide—has selected two exceptional initiatives that were collectively awarded $1M in funding. Dr. Xingxing Zang's innovative work on TOP CAT CAR-T therapy has emerged as a pivotal endeavor, marking a transformative step in addressing rhabdomyosarcoma (RMS), the cancer that took Sebastian's life at the age of 16 – 14 months after his diagnosis.

Project Description:
Rhabdomyosarcoma, a highly aggressive malignant primary muscle cancer, impacts approximately 350 children in the U.S. annually. This formidable disease is characterized by its rapid growth and tendency to spread quickly, often requiring intensive treatment, including surgery, radiation, and chemotherapy. Led by Dr. Xingxing Zang at the Albert

SEBASTIANSTRONG
FOUNDATION

Einstein College of Medicine, this project introduces TOP CAT, a weaponized T-Cell approach that can effectively eliminate various human solid cancers in preclinical studies. T cells are the main killers of cancer cells in the human body. By engineering T cells to recognize and destroy cancer cells, this research represents a promising new direction in immunotherapy. With the support of SebastianStrong Foundation, Dr. Zang teamed with Drs. David Loeb and Allison Martin at Children's Hospital at Montefiore will use TOP CAT to treat human rhabdomyosarcoma, hoping to advance this new immune cell therapy to clinical trials within a few years and benefiting patients with rhabdomyosarcoma.

Impact & Promise:

There have been no significant improvements in survival rates for pediatric patients with RMS since the 1980s. TOP CAT CAR-T therapy embodies the hope for a future where children with rhabdomyosarcoma have access to safer, more effective treatments. As this project progresses towards clinical trials, it highlights the crucial role of innovative research in overcoming the limitations of current cancer therapies. The anticipated impact extends beyond immediate clinical applications, potentially setting new standards for treating various pediatric cancers.